Congressional Testimony on Stem Cells

Statement of Maria C. Freire, Ph.D. Director, National Institutes of Health Office of Technology Transfer before the Senate Appropriations Subcommittee on Labor, Health and Human Services, Education and Related Agencies

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Mr. Chairman and members of the subcommittee, I am Maria Freire, Director of the Office of Technology Transfer at the National Institutes of Health (NIH). I am pleased to appear before you today to address how intellectual property considerations affect basic science and the future development of products for public benefit.

I understand that the subcommittee is particularly interested in how patent rights and commercialization strategies operate in the context of the recent findings on pluripotent stem cells reported by Drs. John Gearhart from Johns Hopkins University and James Thomson from the University of Wisconsin. You have previously heard from a panel of experts, including the Director of NIH, Dr. Harold Varmus, on the scientific implications of these findings. Given the complexity of these issues, it is important to understand how the transfer of federally funded technology from the not-for-profit sector — be it university or Federal laboratory — to the private sector, is accomplished. To do so, I direct you to the successful process established by Congress in the 1980's that governs the commercialization of federally funded biomedical research.

The Bayh-Dole Act, Stevenson-Wydler Technology Innovation Act of 1980, and amendments, including the Federal Technology Transfer Act of 1986 (FTTA)

Nearly twenty years ago, Congress enacted a series of laws that encourage government owned and government funded research laboratories to pursue the commercialization of the results of their research. These laws are the Bayh-Dole Act of 1980, the Stevenson-Wydler Innovation Act of 1980, including one of its amendments, the Federal Technology Transfer Act of 1986 (FTTA). The Bayh-Dole Act addresses intellectual property rights in federally funded grants, contracts and cooperative agreements, while Stevenson-Wydler and the FTTA address intellectual property of government laboratories. The goal of these laws is to promote economic development, enhance U.S. competitiveness and benefit the public by encouraging the commercialization of technologies that might otherwise not be developed into products due to the lack of incentives. Generally, these laws allow government laboratories and the recipients of government funding to elect to retain title to their inventions. They also impose certain obligations: promoting utilization, encouraging commercialization and ensuring public availability of these technologies.

I am pleased to say that these goals have been achieved and expectations have been surpassed. Indeed, in the biomedical arena, the impact of these statutes has been dramatic. Many experts believe that the biotechnology industry was spawned from the close interaction between academia and industry. The Bayh-Dole Act and the FTTA continue to contribute to the global leadership of the U.S. biomedical enterprise. New products developed under this system benefit patients daily and provide hundreds of scientists with the tools required for further discovery in support

of our public health mission. The NIH intramural program alone has over 150 products on the market, including diagnostic kits, vaccines, therapeutic drugs and dozens of antibodies, cell lines and other research tools. Statistics on the remarkable success of university-based technology transfer activities are also available and I have submitted a recent survey for the record.

To accomplish the transfer of technology, universities have relied on authorities granted to them by the Bayh-Dole Act. The Act permits the grantee to retain title to intellectual property developed with federal funds and to license its rights to for-profit entities. Patents provide the right to exclude others from making, using, or selling a new invention for the life of the patent. This is society's reward to the owner for teaching others how to make and use the invention claimed in the patent. In the biomedical field, patents are extremely valuable to companies, particularly small companies. They provide a means of securing investment income by establishing the company's preeminence in a particular area of technology. Parties interested in practicing an invention, in which they have no ownership, may obtain rights to the invention by entering into a licensing agreement with the patent owner. A license is a contract with binding commitments on each party, usually involving compensation. A license does not grant title to the invention. Licenses can be exclusive, when only one party is permitted to benefit from the use of the technology, or non-exclusive, when more than one party is allowed to benefit from such rights.

As this subcommittee well knows, new drugs and vaccines are costly to develop; companies will not invest in further research and development without some promise of future product exclusivity. When Congress gave federal grantees the ability to patent and exclusively license government-funded inventions, the private sector turned its attention toward publicly supported research as a new source of potential products. The value to the public resides in the generation of new drugs, vaccines, and medical devices. These activities have also stimulated economic development and the creation of new jobs in the United States.

The University of Wisconsin provides us with a good example of how the Bayh-Dole Act is implemented. Early work by Dr. Thomson on non-human primates, such as Rhesus monkeys, was federally funded and therefore, the patent obtained on stem cells arising from this work is governed by this Act. In accordance with the law, the invention was disclosed to the NIH, a patent application was filed by the University, through the Wisconsin Alumni Research Foundation (WARF), and WARF licensed the technology to a small company (Geron). Because federal funds were used for this non-human primate work, the government has a non-exclusive, royalty-free right to use the patented cells by or on behalf of the government. This would allow the government laboratories and contractors the right to use the patented cells for further research. In addition, in handling this invention the University must ensure that the goals of the Bayh-Dole Act -- utilization, commercialization, and public availability -- are implemented.

When research is funded entirely by the private sector, the government has no license, and it is strictly a private matter whether, and under what terms, new intellectual property is made available to others for commercial or research purposes. This is the case for the Geron sponsored work conducted by Dr. Gearhart on human pluripotent stem cells derived from fetuses.

It is usually not the existence of a patent that raises concern for the biomedical research community. The concern arises when the patent holder chooses to exercise

its rights through licensing in a manner inconsistent with the advancement of basic research. For example, many new inventions are not final products. The discovery may be a research material or a new method or procedure, primarily useful as the means to conduct further research. Such discoveries are commonly known as research tools. There is little doubt that these research tools may be patentable and that they are of economic value to the holder of these rights. There is also little doubt that the value to society is greatest when such research tools are widely available to scientists.

Mr. Chairman, I cannot emphasize this point strongly enough. Preserving research uses is extremely important to the advancement of science. A license that provides complete exclusivity to a technology that is also a research tool may result in some product development in the short- term, but it will close off opportunities to advance science and develop other products in the long-term. The only way to maximize the benefit to the public is to ensure that both research use and the potential for commercial development are preserved.

The professionals working in the specialized field of biomedical licensing strive to promote a balance between commercial interests and the public interest. In those instances where a research tool can also become a therapeutic product, licenses can be, and are, carefully crafted by scope, application and field to allow use by the research community without destroying a company's commercial incentive to develop the product. Careful licensing that preserves this balance, however, has not always been the case. The NIH has been concerned for some time about the potential adverse effects of restrictive licensing practices on access to research tools. Dr. Varmus convened a national workgroup to study the issue and make recommendations to the NIH. The report of the workgroup is on the NIH web site (www.nih.gov./news/researchtools/index.htm), and NIH expects to publish guidelines for NIH supported investigators this spring, in accordance with the report.

Stem Cell Research

How does this relate to pluripotent stem cells? Pluripotent stem cells provide the research community a springboard to launch numerous inquiries into the most fundamental processes of cellular growth and differentiation that underlie human development. Elucidating these mechanisms provides the foundation for the next generation of biomedical discovery. Such discoveries will be directed toward treatment of human developmental abnormalities, regulation of uncontrolled cellular growth associated with cancer, a source of differentiated cells and tissues for transplantation therapy, and a means to identify new drug targets and test potential therapeutics, among others. Realizing the fullest potential from this new stem cell technology for the American people deserves and requires further inquiry.

Stem cells are a research tool today; hopefully, they will also be developed into therapeutic products in the future. The issuance of patents on these new discoveries by the Patent and Trademark Office may not necessarily have an adverse effect on continuing research, provided that the patent owners devise a licensing strategy that will allow basic research to continue unencumbered while preserving commercial value. We understand that both the Johns Hopkins and Wisconsin licenses to Geron are exclusive at this time, but may allow for the use of these cells by non-profit researchers under certain terms and conditions. These terms and conditions would be set forth in an agreement commonly called a Material Transfer Agreement, or MTA.

MTAs are vehicles used to transfer proprietary materials between and among the for-profit and not-for-profit sectors. While most MTAs are simple, 1 to 2-page agreements, MTAs can sometimes pose problems due to the type of obligations or restrictions imposed by the provider of a material on the recipient. Such obligations can stifle the broad dissemination of new discoveries, slow the technology transfer process and limit future avenues of research and product development. Examples of such obligations include so-called "reach-through" provisions that may: 1) give the provider of a material ownership of new inventions developed by the recipient; 2) require royalty payments by the recipient to the provider on inventions discovered by the recipient that are not covered by the provider's patent; or, 3) require options to exclusive rights to any new intellectual property arising from recipient's use of the material. The NIH has minimal authority with regard to the stem cell patent and patent applications at issue today, and it would be inappropriate for me to try to comment on specific terms and conditions that may be imposed by these parties under the MTAs contemplated.

At NIH, our view is that conditions imposed by patent owners - whether in a license or an MTA - can be crafted to ensure both research uses and commercial development. For example, our strategy is to negotiate non-exclusive licenses whenever possible. This allows more than one company to develop products using a particular technology, products that may ultimately compete with each other in the marketplace. We recognize that companies need an exclusive market to offset the risk, time, and expense of developing biomedical diagnostic or therapeutic products. However, companies do not necessarily need to achieve that position solely by exclusively licensing a government technology used to develop the product. Instead, companies are frequently able to add their own proprietary technologies to the invention licensed from the government to ultimately achieve some level of uniqueness and exclusivity for the final product.

If non-exclusive licensing does not provide enough incentive for the company to develop a product, and it often does not for a potential therapeutic application, NIH will award exclusivity for specific indications or fields of use, based on the license applicant's commercial development plans at the time of the application. NIH also requires exclusive licensees to grant sublicenses to broaden the development possibilities when necessary for the public health. Finally, NIH insists on the continuing unencumbered availability of the licensed technology to not-for-profit scientific community for further research.

Experience over the last 20 years has shown that to maximize public health benefit, the balance between exclusivity and access must be carefully maintained and research uses of new technologies must be preserved. These concepts form the basis for the licensing policies of the NIH, as well as for the proposed guidelines for our grantees mentioned above.

Summary

Congress has enacted legislation for recipients of federal funding that encourages the utilization, commercialization and public availability of federally funded inventions. Grantees have exercised broad discretion and appropriately seek to achieve these goals through the patenting and licensing of new inventions that arise through the use of federal funds. If the research is entirely funded by the private sector, the government has no license and is not involved in patenting or licensing decisions.

Exclusive licensing, without regard to research uses, can impede rather than enhance utilization and public availability of certain types of inventions, such as research tools. Strategic licensing can alleviate potential problems. Indeed, many grantees provide for the continuing availability of exclusively licensed subject matter to researchers in order to ensure progress of biomedical research. The NIH has urged, and will continue to urge, patent owners and exclusive licensees to ensure continuing availability under terms that do not limit basic research or encumber future products.

Mr. Chairman, I am grateful to you for providing a forum to present information about the effects of patents and licenses on this promising new area of science and medicine. I would be pleased to answer any questions you may have.